25 February 2019
EMA/833241/2018

Public summary of opinion on orphan designation

Sodium 2-hydroxylinoleate for the treatment of biliary tract cancer

On 14 December 2018, orphan designation (EU/3/18/2121) was granted by the European Commission to Ability Pharmaceuticals SL, Spain, for sodium 2-hydroxylinoleate (also known as ABTL0812) for the treatment of biliary tract cancer.

What is biliary tract cancer?

Biliary tract cancer is cancer of the bile ducts and gallbladder. These are parts of the digestive system that transport and store bile, a fluid produced by the liver and released into the intestines after a meal to help digest fats. The cancer is characterised by various features such as abnormal liver function tests, pain in the belly, yellowish discoloration of the skin and weight loss.

Biliary tract cancer is a long-term debilitating and life-threatening disease due to liver failure and problems caused when the cancer blocks the bile ducts.

What is the estimated number of patients affected by the condition?

At the time of designation, biliary tract cancer affected approximately 1.5 in 10,000 people in the European Union (EU). This was equivalent to a total of around 78,000 people*, and is below the ceiling for orphan designation, which is 5 people in 10,000. This is based on the information provided by the sponsor and the knowledge of the Committee for Orphan Medicinal Products (COMP).

What treatments are available?

At the time of designation, no satisfactory methods were authorised in the EU for the treatment of biliary tract cancer. Some patients with early disease could undergo surgery to remove the cancer. Other treatments included chemotherapy medicines (medicines to treat cancer), although these were not authorised for biliary tract cancer.

*Disclaimer: For the purpose of the designation, the number of patients affected by the condition is estimated and assessed on the basis of data from the European Union (EU 28), Norway, Iceland and Liechtenstein. This represents a population of 517,400,000 (Eurostat 2018).

30 Churchill Place ● Canary Wharf ● London E14 5EU ● United Kingdom
Telephone +44 (0)20 3660 6000 **Facsimile** +44 (0)20 3660 5555
Send a question via our website www.ema.europa.eu/contact

How is this medicine expected to work?

This medicine is expected to work by reducing the activity of several proteins in the 'Akt/mTOR signalling pathway'. This is a mechanism within cells which is important in regulating their growth and survival. In many cancers, including biliary tract cancer, this pathway is overactive, allowing the cancer cells to grow uncontrollably. By reducing the activity of this pathway, the medicine is expected to slow down the progression of the cancer.

What is the stage of development of this medicine?

The effects of the medicine have been evaluated in experimental models.

At the time of submission of the application for orphan designation, no clinical trials with the medicine in patients with biliary tract cancer were ongoing.

At the time of submission, the medicine was not authorised anywhere in the EU for biliary tract cancer or designated as an orphan medicinal product elsewhere for this condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 8 November 2018 recommending the granting of this designation.

_____

Opinions on orphan medicinal product designations are based on the following three criteria:

- the seriousness of the condition;
- the existence of alternative methods of diagnosis, prevention or treatment;
- either the rarity of the condition (affecting not more than 5 in 10,000 people in the EU) or insufficient returns on investment.

Designated orphan medicinal products are products that are still under investigation and are considered for orphan designation on the basis of potential activity. An orphan designation is not a marketing authorisation. As a consequence, demonstration of quality, safety and efficacy is necessary before a product can be granted a marketing authorisation.

For more information

Sponsor's contact details:

Contact details of the current sponsor for this orphan designation can be found on the EMA website.

For contact details of patients' organisations whose activities are targeted at rare diseases see:

- Orphanet, a database containing information on rare diseases, which includes a directory of patients' organisations registered in Europe;
- European Organisation for Rare Diseases (EURORDIS), a non-governmental alliance of patient organisations and individuals active in the field of rare diseases.

Translations of the active ingredient and indication in all official EU languages[1], Norwegian and Icelandic

Language	Active ingredient	Indication
English	Sodium 2-hydroxylinoleate	Treatment of biliary tract cancer
Bulgarian	Натрий 2-хидроксилинолеат	Лечение на рак на жлъчните пътища
Croatian	Natrijev 2-hidroksilinoleat	Liječenje raka bilijarnog trakta
Czech	2-hydroxilinoleát sodný	Léčba karcinomu žlučových cest
Danish	Natrium 2-hydroxylinolat	Behandling af galdegangscancer
Dutch	2-hydroxilinoleaat natrium	Behandeling van galweg kanker
Estonian	Naatrium 2-hüdroksülinolaat	Sapiteede kasvaja ravi
Finnish	Natrium 2-hydroksilinolaatti	Sappiteiden syövän hoito
French	2-hydroxylinoléate de sodium	Traitement du cancer des voies biliaires
German	Natrium 2-hydroxilinolat	Behandlung von Tumoren der Gallenwege
Greek	2-υδροξυλινελαϊκο νάτριο	Θεραπεία του καρκίνου της χοληφόρου οδού
Hungarian	Nátrium 2-hidroxilinolát	Epeúti rák kezelése
Italian	2-idrossilinoleato di sodio	Trattamento del carcinoma delle viebiliari
Latvian	Nātrija 2-hidroksilinoleāts	Žultsvadu sistēmas vēža ārstēšana
Lithuanian	Natrio 2-hidroksilinoleatas	Tulžies latakų vėžio gydymas
Maltese	Sodium 2-hydroxylinoleate	Kura tal-kanċer tal-apparat tal-bili
Polish	2-hydroksylinolean sodu	Leczenie raka dróg żółciowych
Portuguese	2-hidroxilinoleato de sódio	Tratamento da neoplasia das vias biliares
Romanian	2-hidroxilinoleat de sodiu	Tratamentul cancerului de căi biliare
Slovak	2-hydroxilinoleát sodný	Liečba karcinómu žlčových ciest
Slovenian	Natrijev 2-hidroksilinolat	Zdravljenje raka žolčnih vodov
Spanish	2-hidroxilonoleato sódico	Tratamiento del cáncer del árbol biliar
Swedish	Natrium 2-hydroxilinolat	Behandling av gallvägscancer
Norwegian	Natrium-2-hydroksylinoelat	Behandling av gallegangskreft
Icelandic	Natríum 2-hýdroxýlínólati	Meðferð við krabbameini í gallvegum

[1] At the time of designation